Santhera Pharmaceuticals

Developing medicines to meet the needs of patients living with rare diseases

Presentation by Dario Eklund, CEO

Sep 2023

Version S

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Presentation Overview

(1) Santhera in a snapshot

(2) Duchenne muscular dystrophy (DMD) and the need for a better foundational therapy

(3) Vamorolone and commercial strategy

(4) Financial status


Headquarters near Basel, Switzerland

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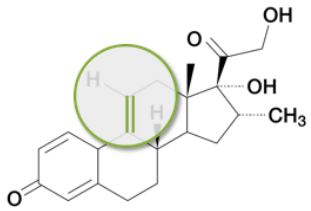
Commercial stage company in attractive DMD market

- **SIX Swiss Exchange listed company (SANN)**
- **Lead asset vamorolone in DMD (and beyond)**
  - Safer alternative to corticosteroids
  - Regulatory decisions in Q4-2023 (US, EU, UK)
    - Recent Catalyst US deal valued at up to USD 231 million plus royalties
- **Cash to execute own EU commercial strategy into 2025**

SIX Swiss Exchange



EUROPEAN MEDICINES AGENCY

FDA

Catalyst pharmaceuticals

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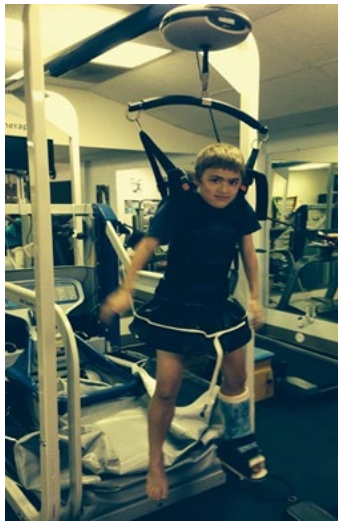
Duchenne muscular dystrophy (DMD)

- **Genetic disease causing progressive muscle weakness**
  - Loss of ambulation in early teenage years
  - Respiratory failure and cardiac complications
  - Life expectancy in the late twenties
  - 30 – 35,000 patients in US and EU, combined
  - Corticosteroids standard of care
- **Current therapies with intrinsic limitations**
  - Exon skipping drugs
  - Micro-dystrophin gene therapy



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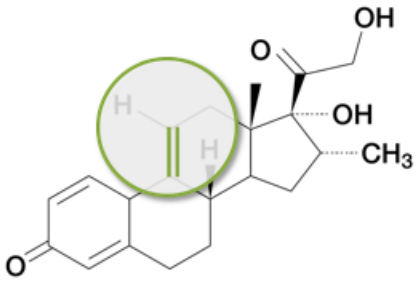
Vamorolone can fill the need for a better foundational therapy in DMD

ESTABLISHED EFFICACY OF STEROIDS	SAFETY ISSUES WITH STEROIDS	VAMOROLONE OFFERING
ESTABLISHED FOUNDATIONAL THERAPY	TOO LATE TOO LITTLE TOO SHORT	NEW DISSOCIATIVE STEROID CLASS



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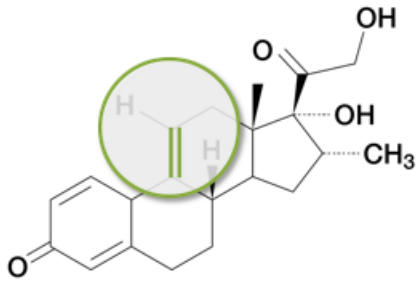
Vamorolone dissociative properties

Subtle but impactful difference in chemical structure separates vamorolone from classical steroids[1-5]



Signature double bond impacts receptor binding and alters enzyme and membrane interactions

Like corticosteroids, efficacy maintained by potent anti-inflammatory action

- Retained inhibition of NF-κB pro-inflammatory transcription factor

Unlike corticosteroids, potential for reduction of steroid-associated side effects

- Less activation of genes related to side effects
- Not a substrate of hydroxysteroid dehydrogenase
- Potent mineralocorticoid antagonist (eplerenone-like)
- Membrane stabilizer

NF-κB=nuclear factor kappa B., 1. Smith et al. PLOS Medicine. (2020); 2. Dang et al. MDA Abstr. #47 (2021) 3. Guglieri M Poster EP 524 WMS 2021, 4. Heier CR, et al. EMBO Mol Med. 2013;5:1569-1585, 5. Liu X, Proc Natl Acad Sci U S A. 2020 Sep 29;117(39)

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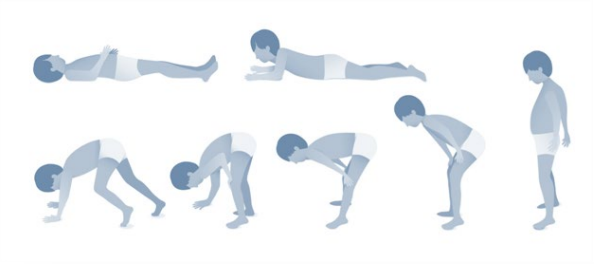
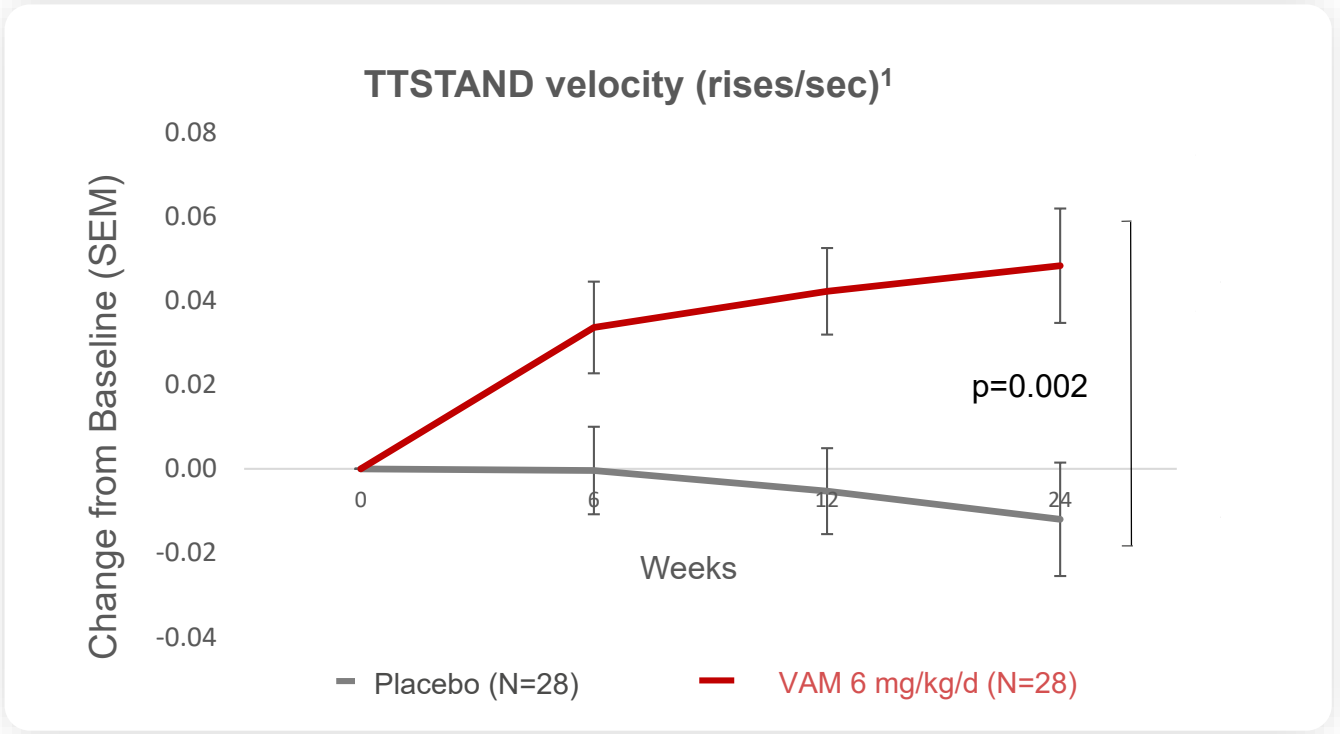
Primary endpoint was met in pivotal clinical trial (VISION-DMD)



Rise time (sec)[1,2]	BL	w 24	% Change
VAM 6 mg/kg/d	6.0	4.6	**- 23%**
Placebo	5.4	5.5	+ 2%



1. mITT-1: modified intention to treat population from period 1, MMRM estimates of changes from baseline (BL),
2. Press Release June 1, 2021, descriptive statistics

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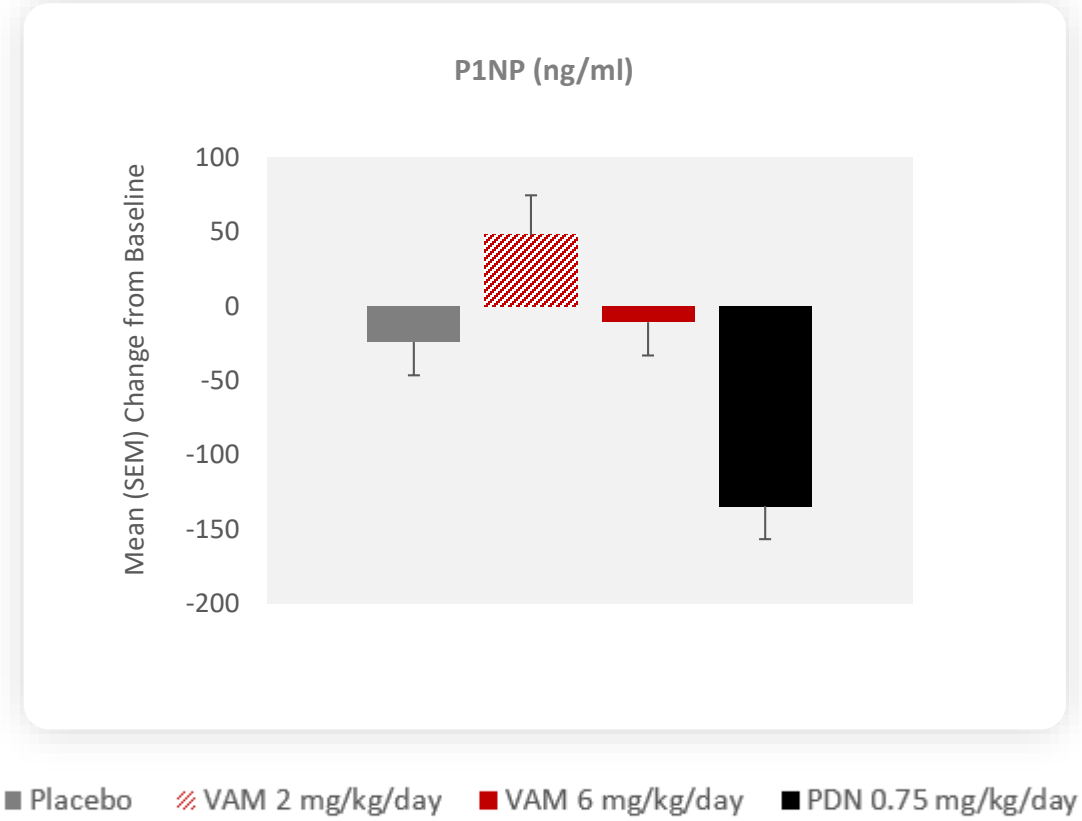
Vamorolone has no negative impact on bone biomarkers

Example shown for biomarker of bone formation (VISION-DMD at 24 weeks)



Data on File : VAM-2021-007, PDN, prednisone; SEM, standard error of mean; VAM, vamorolone. P1NP, procollagen type 1 N-terminal pro-peptide. Safety population (SAF-1) at 24 weeks

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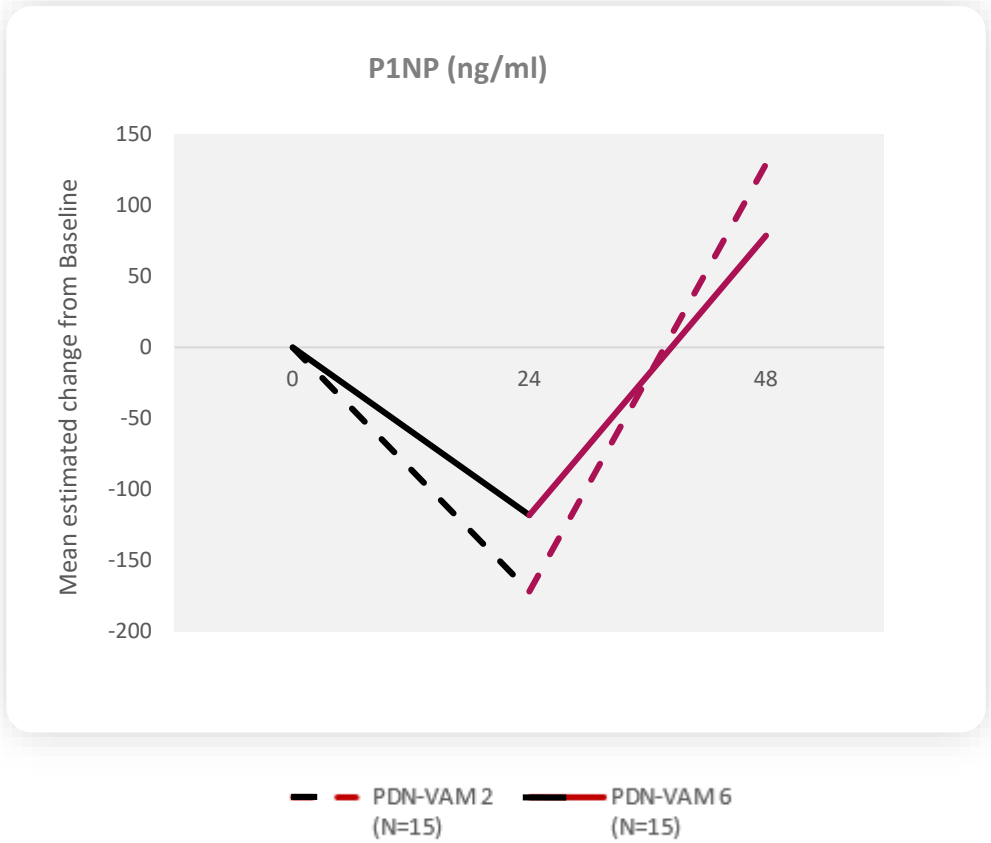
Vamorolone leads to biomarker recovery after switch from prednisone

Example shown for biomarker of bone formation (VISION-DMD)



Data from VISION-DMD Pivotal Study on File 2022, PDN, prednisone; VAM, vamorolone.
P1NP, procollagen type 1 N-terminal pro-peptide. Safety population (SAF-2), change from baseline to week 48

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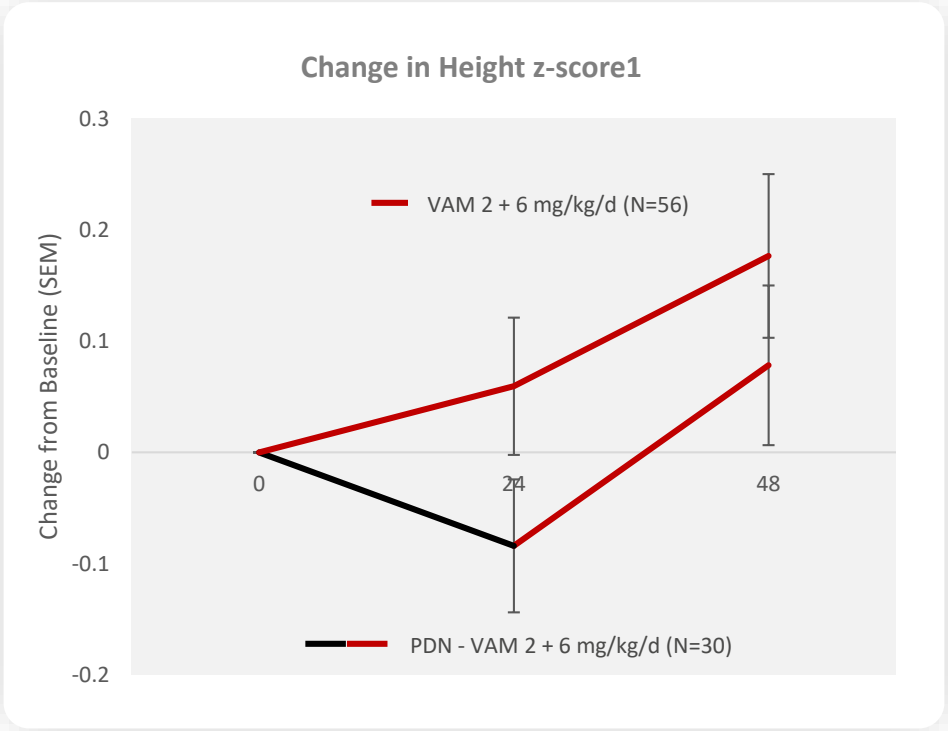
Switching from prednisone to vamorolone recovers normal growth

Growth trajectory (VISION-DMD)



Data from VISION-DMD Pivotal Study: Safety Population 2 (SAF-2); PDN – Prednisone 0.75 mg/kg/d; PDN-VAM: growth trajectory (z-score) compared for prednisone in Period 1 and vamorolone (2 + 6 mg/kg/d) in Period 2; All doses daily; MMRM estimates of changes from baseline; Mah et al; ePoster LB.08 WMS 2021

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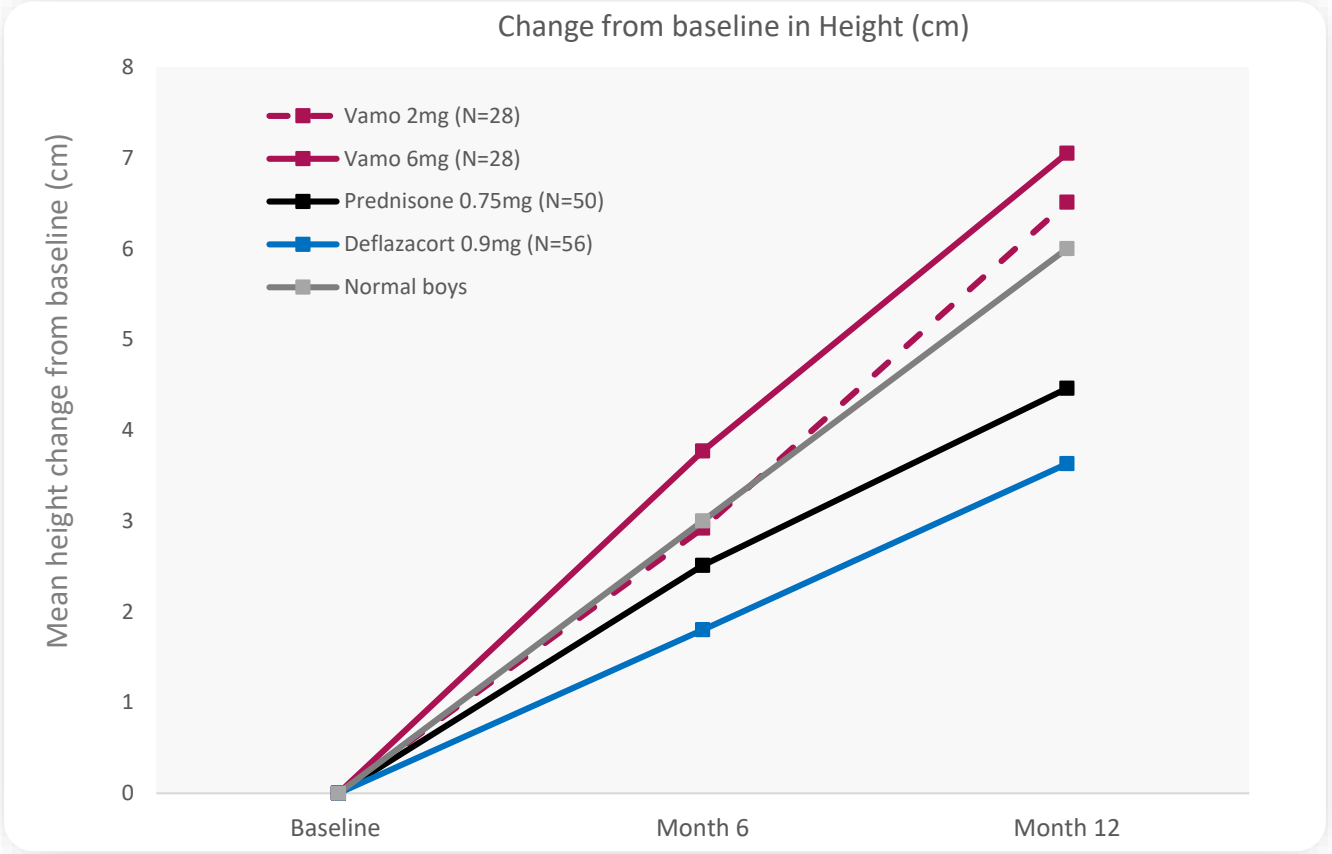
Vamorolone unlike other steroids preserves normal, long-term growth

Growth trajectory VISION-DMD vs FOR-DMD (natural history)



Vision-DMD Data on File 2022; Poster 71 WMS 2022; All doses mg/kg, daily.
Pre-specified propensity matched comparison from VISION-DMD vs FOR-DMD and comparison of VBP15-LTE vs FOR-DMD

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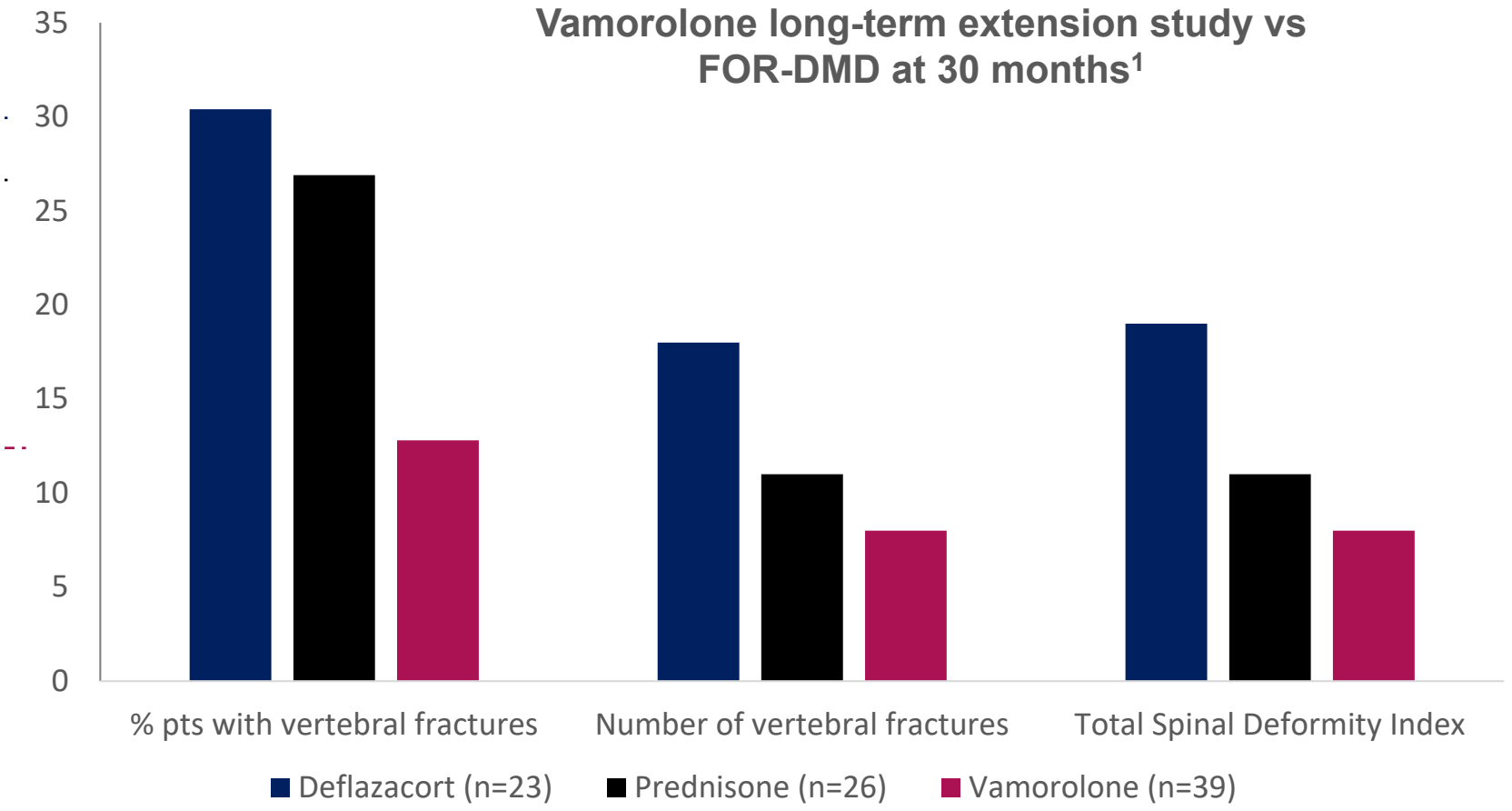
Vamorolone leads to fewer and less severe spinal fractures

Reduction of number of patients with vertebral fractures by approx. 50%



1: https://www.santhera.com FP03-WMS_poster_20_August_2022.pdf Spinal Deformity Index (SDI): sum of the Genant Grades from T4 to L4, and therefore, is the composite of both fracture number and severity

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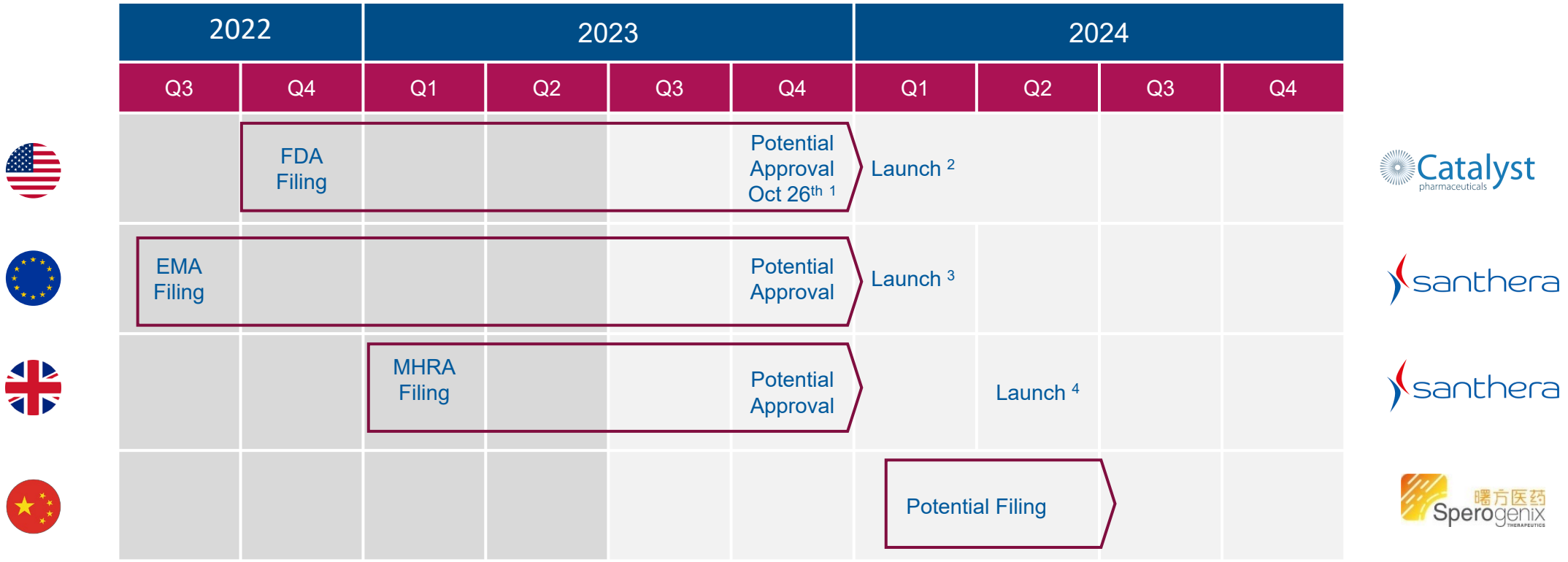
Summary of value proposition for vamorolone in DMD

- **Durable efficacy comparable to standard of care**
- **Preserved bone health (unlike deleterious effect of standard steroids)**
- **Improved safety profile compared to prednisone evident at 24 weeks**
- **Long-term treatment profile being further explored in LTE and EAPs**

LTE: Long-term extension study; EAP: Early access program

Potential approvals in Q4-2023 allow Q1-2024 launches in US and EU



Orphan drug exclusivity in US (7 years) and Europe (12 years incl. ped. extension)

Patent protection at least until 2040 (US) and 2035 (EU)

1: FDA decision expected at so-called PDUFA date set to October 26th, 2023 2: Expected through partner Catalyst
3: Staggered launch starting with Germany;4: Launch review by NICE (National Institute for Health and Care Excellence) evaluation
EU orphan protection 10 years plus pediatric 2-year extension; EU IP Including 5-year Supplementary Patent Certificate (SPC)

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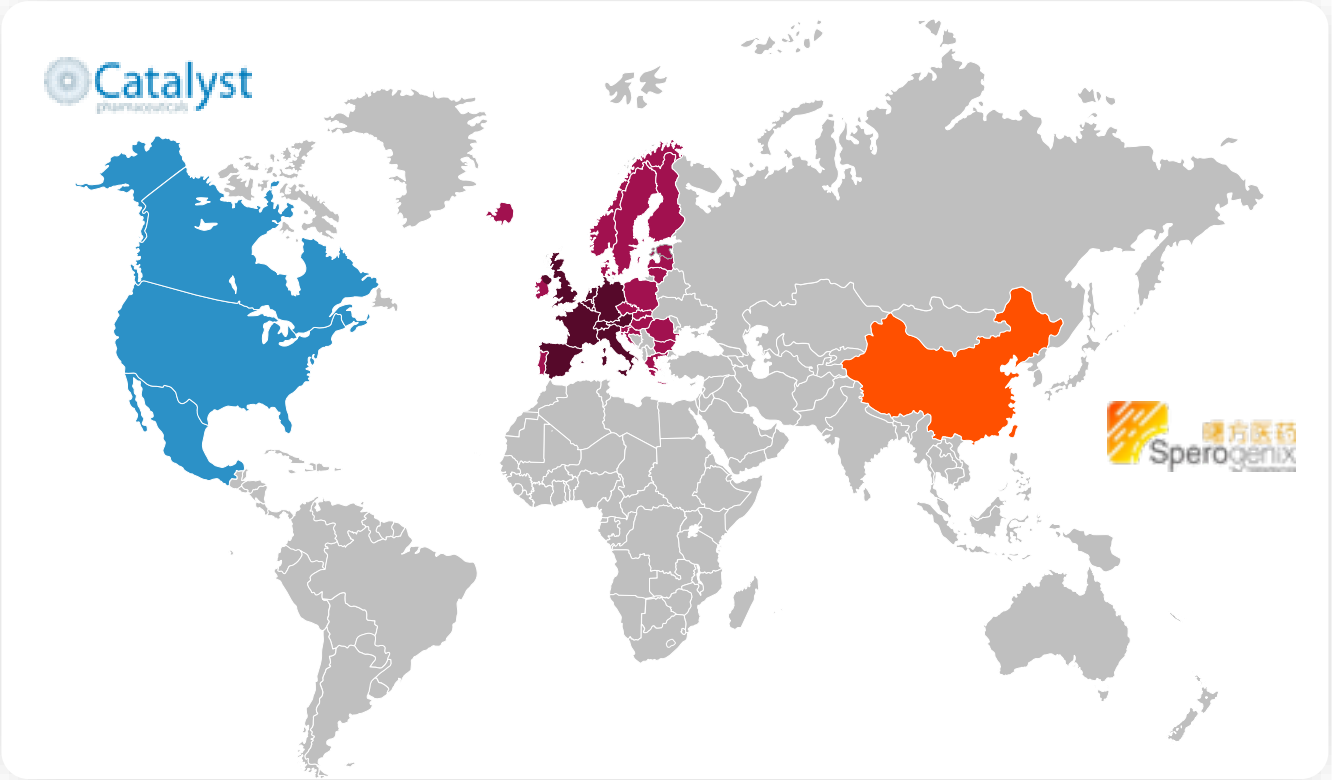
Santhera holds global rights to vamorolone in all indications

- **North America**
  - Partnership with Catalyst (NA)
- **China**
  - Partnership with Sperogenix (CN)
- **Europe**
  - Own commercialization (next slide)
- **Rest of world**
  - Further out-licensing opportunities



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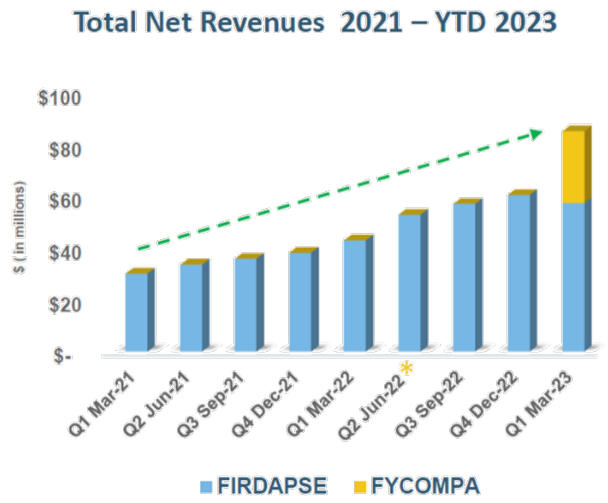
Catalyst is our commercial partner of choice for North America

- **Founded 2002, IPO 2006**
- **Market capitalization USD 1.4 billion as of June 22, 2023 (CPRX, Nasdaq)**
- **Proven commercial capabilities: targeted total revenues 2023 of USD 375 – 385 million**
  - Two marketed drugs, Firdapse (amifampridine) and Fycompa (perampanel)
  - **Focus**
    - neurological disorders
    - expand portfolio in rare and orphan diseases
  - **Strong financial position for growth***
    - total revenues of USD 85.4 million for Q1-2023
    - strong cash generation capability
    - cash of USD 148 million (Q1-2023)

Strong Financial Position Underscores Successful Execution of Strategic Initiatives

(in millions)

Q1 23 Results	
Cash Position	$148.2
Record Net Product Revenue	$85.3
FY 2023 Projections	
Total Net Revenue Estimates	$375 - $385
FIRDAPSE Net Product Revenue	$245 - $255
FYCOMPA Net Product Revenue	$130
Non-GAAP Net Income	$195 - $205



* Catalyst Pharmaceuticals Corporate Presentation June 2nd, 2023

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Catalyst Pharmaceuticals:

Transformative deal valued up to USD 231 million + royalties

- **Upfront of USD 75 million represents more than Santhera's market capitalization pre-signing**
  - Provides funding for EU launch and for healthy balance sheet through repayment of short-term debt
  - Additional equity investment of USD 15 million funds future development

- **Santhera exploits vamorolone potential outside of North America while developing & co-funding future global indications together with Catalyst**

- **Catalyst commits USD 36 million for FDA approval milestone (of which Santhera retains USD 10 million)**

- **Santhera continues to participate in the success of vamorolone in North America**
  - Up to USD 105 million in potential sales milestones
  - Up to low-teen % annual royalty payments to Santhera
  - Catalyst pays third-party royalties related to North American net sales

Santhera commercial launch in key European geographies

- **Early access programs starting in Q4-2023 in FR and UK**
- **First launch in Germany in early Q1-2024**
- **Commercialization in core Western markets by Santhera**
  - Lean commercial organization with up to 60 incremental FTEs
  - Peak sales of EUR >150 million in Santhera territory in DMD alone
- **Commercialization through distributors outside core markets**
  - Additional revenue from partners

EAMS: Early Access to Medicines Scheme; AAP: Autorisation d'Accès Précoce (Early access authorisation)

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Vamorolone – a pipeline in a product with multi-indication potential

- **Focus on patient population benefiting from a prolonged and safer steroid treatment**
- **Ongoing selection process identified various therapeutic areas**
  - Internal research supported by *Back Bay Life Science Advisors, KPMG* and *tranScrip*
- **Candidate indications for development will be prioritized over the coming months together with partner Catalyst**

Therapeutic Area	Indication
Neurology	Becker Muscular Dystrophy*
	Myasthenia Gravis Ocular*
	Chronic Inflammatory Demyelinating Polyneuropathy
Pulmonology	Sarcoidosis*
	Idiopathic Interstitial Pneumonitis
Nephrology	Frequently Relapsing Nephrotic Syndrome*
	Membranous Nephropathy
Rheumatology	Dermatomyositis
	Juvenile Rheumatoid Arthritis
	Polymyalgia Rheumatica
Hematology	Genetic or Acquired Anemia
Hepatology	Autoimmune Hepatitis Type 2

* Indications with most advanced analyses

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Santhera financial status

Santhera Pharmaceuticals is listed on the Swiss Stock Exchange SIX: Ticker SANN

- **Key figures (CHF million as of June 30, 2023*)**
  - Net (loss) for the period (23.3)
  - Cash (used) in operations (15.5)
  - Cash & cash equivalents 1.7
  - Debt outstanding (maturity 2024) ** (49.1)
  - Shareholders` equity (42.8)

- **Capital structure (as of August 31, 2023)**
  - Basic shares outstanding 12.6 million
  - Market capitalization CHF 110 million (per share CHF 8.74)
  - Major shareholders Catalyst (11.2%) and Idorsia (10.3%)
  - Research coverage by H.C. Wainwright and valuationLAB

- **Recent transaction**
  - 07-2023: Vamorolone US licensing to Catalyst
    Raxone/idebenone divestment to Chiesi

- **Cash runway**
  - Into 2025 incl commercial EU infrastructure & launch

- **Upcoming milestones vamorolone**
  - Q4-2023: CHMP (EU), MHRA (UK) and
    FDA decision (US PDUFA Oct 26th)
  - Q4-2023: Early access programs in UK and France
  - Q1-2024: Commercial launch by Catalyst (US) and
    Santhera (EU)

*All amounts unaudited consolidated IFRS ; ** Includes Listed Convertible Bond Maturing Aug-24 CHF 13.5m

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Santhera is well positioned for future growth

Future potential for vamorolone as pipeline within a product not factored in current valuation

- **Healthy balance sheet with low debt and sufficient cash for own commercialization in core EU markets**

- **Non-dilutive income via own EU sales, licensing income and additional opportunities for partnering**
  - DMD market is well defined across global territories with a need for better foundational therapy
  - EUR >150 Mio peak sales in Santhera European territory with vamorolone in first indication DMD
  - Strong partner Catalyst for US and worldwide opportunities beyond existing China partnership with Sperogenix
  - International patent protection at least until 2040 (US) and 2035 (EU)

- **Potential beyond DMD for vamorolone as replacement for chronic steroid treatment**
  - Additional indication development to be conducted jointly with Catalyst Pharmaceuticals
  - Shortlist of indication further prioritized in coming months

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Developing medicines to meet the needs of patients living with rare diseases

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